

SEQUENCE LISTING

<110> Hildinger, Markus

<120> Decreasing gene expression in a mammalian subject in vivo via AAV-mediated RNAi expression cassette transfer

<130> 2

<140> US 10/604,340

<141> 2003-07-13

<160> 12

<170> PatentIn version 3.2

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<212> DNA

<213> Artificial

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<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/2 CMV luciferase

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expression cassette; referred to as AAV2/5 CMV luciferase

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aga	ttc	tgc	cat	gcc	aga	gat	cct	att	ttt	ggc	aat	caa	atc	att	ccg		1926
Arg	Phe	Ser	His	Ala	Arg	Asp	Pro	Ile	Phe	Gly	Asn	Gln	Ile	Ile	Pro		
		220					225					230					
gat	act	gcg	att	tta	agt	gtt	gtt	cca	ttc	cat	cac	ggc	ttt	gga	atg		1974
Asp	Thr	Ala	Ile	Leu	Ser	Val	Val	Pro	Phe	His	His	Gly	Phe	Gly	Met		
		235				240					245						
ttt	act	aca	ctc	gga	tat	ttg	ata	tgt	gga	ttt	oga	gtc	gtc	tta	atg		2022
Phe	Thr	Thr	Leu	Gly	Tyr	Leu	Ile	Cys	Gly	Phe	Arg	Val	Val	Leu	Met		
		250			255				260					265			
tat	aga	ttt	gaa	gaa	gag	ctg	ttt	ctg	agg	agg	ott	cag	gat	tac	aag		2070
Tyr	Arg	Phe	Glu	Glu	Glu	Leu	Phe	Leu	Arg	Ser	Leu	Gln	Asp	Tyr	Lys		
			270					275					280				
att	caa	agt	gcg	ctg	ctg	gtg	cca	acc	cta	ttc	tcc	ttc	ttc	gcc	aaa		2118
Ile	Gln	Ser	Ala	Leu	Leu	Val	Pro	Thr	Leu	Phe	Ser	Phe	Phe	Ala	Lys		
			285					290					295				

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agg ttc cat ctg cca ggt atc agg caa gga tat ggg ctc act gag aot Arg Phe His Leu Pro Gly Ile Arg Gln Gly Tyr Gly Leu Thr Glu Thr 330 335 340 345	2262
aca tca gct att ctg att aca ccc gag ggg gat gat aaa ccg ggc gog Thr Ser Ala Ile Leu Ile Thr Pro Glu Gly Asp Asp Lys Pro Gly Ala 350 355 360	2310
gtc ggt aaa gtt gtt cca ttt ttt gaa gcg aag gtt gtg gat ctg gat Val Gly Lys Val Val Pro Phe Phe Glu Ala Lys Val Val Asp Leu Asp 365 370 375	2358
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gac gat gac gcc ggt gaa ctt ccc gcc gcc gtt gtt gtt ttg gag cac Asp Asp Asp Ala Gly Glu Leu Pro Ala Ala Val Val Val Leu Glu His 475 480 485	2694
gga aag acg atg acg gaa aaa gag atc gtg gat tac gtc gcc agt caa Gly Lys Thr Met Thr Glu Lys Glu Ile Val Asp Tyr Val Ala Ser Gln 490 495 500 505	2742
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 Ile Leu Ile Lys Ala Lys Lys Gly Gly Lys Ile Ala Val
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<211> 550
<212> PRT
<213> Artificial

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<220>
<223> Synthetic Construct

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<400> 4

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```

```

Leu Glu Asp Gly Thr Ala Gly Glu Gln Leu His Lys Ala Met Lys Arg
20          25          30

```

```

Tyr Ala Leu Val Pro Gly Thr Ile Ala Phe Thr Asp Ala His Ile Glu
35          40          45

```

```

Val Asp Ile Thr Tyr Ala Glu Tyr Phe Glu Met Ser Val Arg Leu Ala
50          55          60

```

```

Glu Ala Met Lys Arg Tyr Gly Leu Asn Thr Asn His Arg Ile Val Val
65          70          75          80

```

```

Cys Ser Glu Asn Ser Leu Gln Phe Phe Met Pro Val Leu Gly Ala Leu
85          90          95

```

```

Phe Ile Gly Val Ala Val Ala Pro Ala Asn Asp Ile Tyr Asn Glu Arg
100          105          110

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```

Glu Leu Leu Asn Ser Met Gly Ile Ser Gln Pro Thr Val Val Phe Val
115          120          125

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Ser Lys Lys Gly Leu Gln Lys Ile Leu Asn Val Gln Lys Lys Leu Pro
 130 135 140

Ile Ile Gln Lys Ile Ile Ile Met Asp Ser Lys Thr Asp Tyr Gln Gly
 145 150 155 160

Phe Gln Ser Met Tyr Thr Phe Val Thr Ser His Leu Pro Pro Gly Phe
 165 170 175

Asn Glu Tyr Asp Phe Val Pro Glu Ser Phe Asp Arg Asp Lys Thr Ile
 180 185 190

Ala Leu Ile Met Asn Ser Ser Gly Ser Thr Gly Leu Pro Lys Gly Val
 195 200 205

Ala Leu Pro His Arg Thr Ala Cys Val Arg Phe Ser His Ala Arg Asp
 210 215 220

Pro Ile Phe Gly Asn Gln Ile Ile Pro Asp Thr Ala Ile Leu Ser Val
 225 230 235 240

Val Pro Phe His His Gly Phe Gly Met Phe Thr Thr Leu Gly Tyr Leu
 245 250 255

Ile Cys Gly Phe Arg Val Val Leu Met Tyr Arg Phe Glu Glu Glu Leu
 260 265 270

Phe Leu Arg Ser Leu Gln Asp Tyr Lys Ile Gln Ser Ala Leu Leu Val
 275 280 285

Pro Thr Leu Phe Ser Phe Phe Ala Lys Ser Thr Leu Ile Asp Lys Tyr
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Asp Leu Ser Asn Leu His Glu Ile Ala Ser Gly Gly Ala Pro Leu Ser
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Lys Glu Val Gly Glu Ala Val Ala Lys Arg Phe His Leu Pro Gly Ile
 325 330 335

Arg Gln Gly Tyr Gly Leu Thr Glu Thr Thr Ser Ala Ile Leu Ile Thr
 340 345 350

Pro Glu Gly Asp Asp Lys Pro Gly Ala Val Gly Lys Val Val Pro Phe
355 360 365

Phe Glu Ala Lys Val Val Asp Leu Asp Thr Gly Lys Thr Leu Gly Val
370 375 380

Asn Gln Arg Gly Glu Leu Cys Val Arg Gly Pro Met Ile Met Ser Gly
385 390 395 400

Tyr Val Asn Asn Pro Glu Ala Thr Asn Ala Leu Ile Asp Lys Asp Gly
405 410 415

Trp Leu His Ser Gly Asp Ile Ala Tyr Trp Asp Glu Asp Glu His Phe
420 425 430

Phe Ile Val Asp Arg Leu Lys Ser Leu Ile Lys Tyr Lys Gly Tyr Gln
435 440 445

Val Ala Pro Ala Glu Leu Glu Ser Ile Leu Leu Gln His Pro Asn Ile
450 455 460

Phe Asp Ala Gly Val Ala Gly Leu Pro Asp Asp Asp Ala Gly Glu Leu
465 470 475 480

Pro Ala Ala Val Val Val Leu Glu His Gly Lys Thr Met Thr Glu Lys
485 490 495

Glu Ile Val Asp Tyr Val Ala Ser Gln Val Thr Thr Ala Lys Lys Leu
500 505 510

Arg Gly Gly Val Val Phe Val Asp Glu Val Pro Lys Gly Leu Thr Gly
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Lys Leu Asp Ala Arg Lys Ile Arg Glu Ile Leu Ile Lys Ala Lys Lys
530 535 540

Gly Gly Lys Ile Ala Val
545 550

<210> 5
 <211> 3618
 <212> DNA
 <213> Artificial

<220>
 <223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6 lucRI-1a

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<210> 6
 <211> 3920
 <212> DNA
 <213> Artificial

<220>
 <223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6 lucRI-1b

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<210> 7
<211> 3923
<212> DNA
<213> Artificial

<220>
<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6/U6 lucRIU6-3

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<210> 8
<211> 3589
<212> DNA
<213> Artificial

<220>
<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6 lucRI-4(sense)

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<210> 9
<211> 3589
<212> DNA
<213> Artificial

<220>
<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6 lucRI-4(antisense)

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<211> 3617
<212> DNA
<213> Artificial

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<220>
<223> sequence for recombinant adeno-associated viral vector, including
        plasmid backbone, with AAV2 internal terminal repeats that flank
        expression cassette; referred to as AAV2/2 U6 eGFPRI-1a

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<210> 11
<211> 3787
<212> DNA
<213> Artificial

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<220>
<223> sequence for recombinant adeno-associated viral vector, including
        plasmid backbone, with AAV2 internal terminal repeats that flank
        expression cassette; referred to as AAV2/5 pol1 lucR1

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<210> 12
<211> 3941
<212> DNA
<213> Artificial

<220>
<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6/U6 lucRI-2

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